NIHR | Birmingham Biomedical Research Centre

Patient Reported Outcome Data Studies

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BRC & CRF Inclusion Conference

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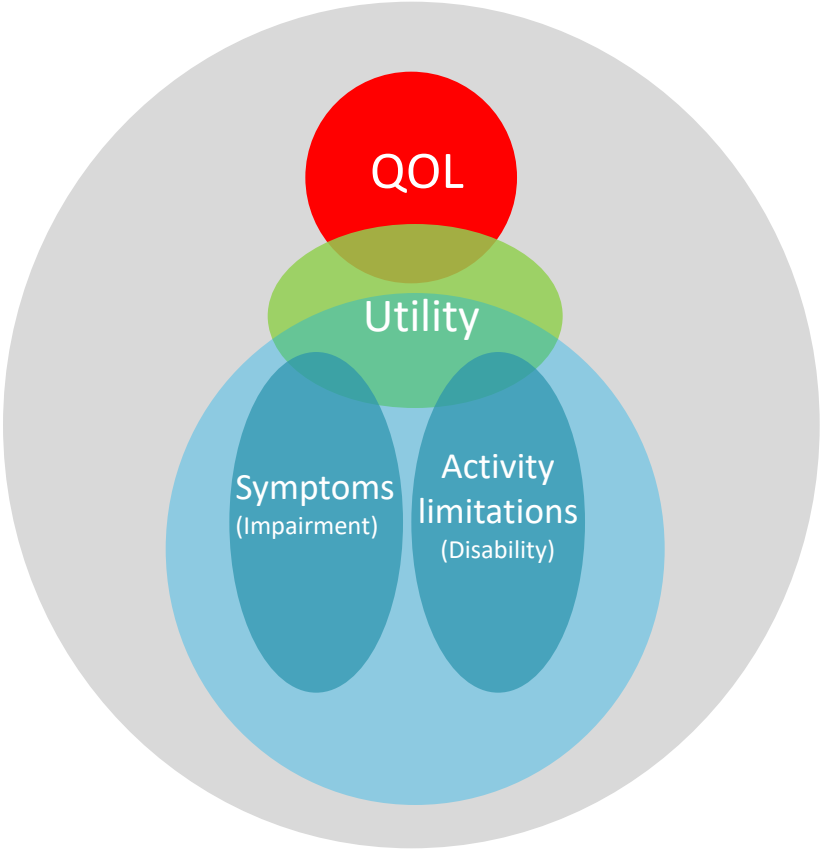
What are patient-reported outcomes?

- "A measurement based on a report that comes **directly from the patient… without amendment or interpretation…** by a clinician or anyone else"[1]
- Concepts known only to the patient
- Rating scales, counting of events, daily diaries
- Generic/disease-specific measures

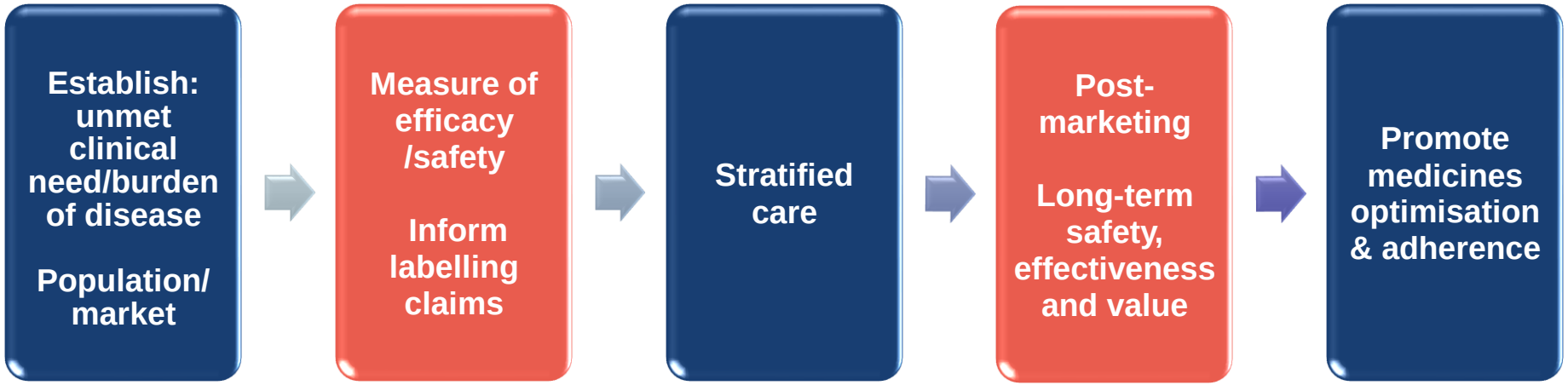


Adapted from McKenna SP. 2011[2]

Why assess PROs in clinical trials?

- Patient-centric
- Assess efficacy or effectiveness
- Inform future patient choice and consent
- Prognostic significance
- Safety endpoints
- Discriminate between therapies in a crowded market
- Inform labelling claims and health policy

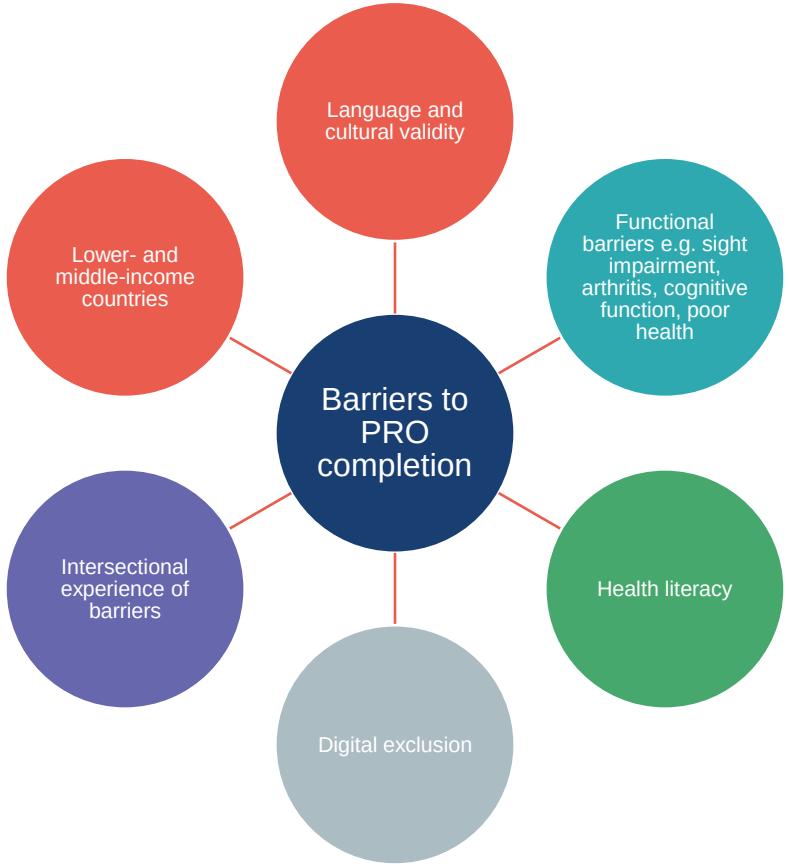
PROs in the Drug Development Process

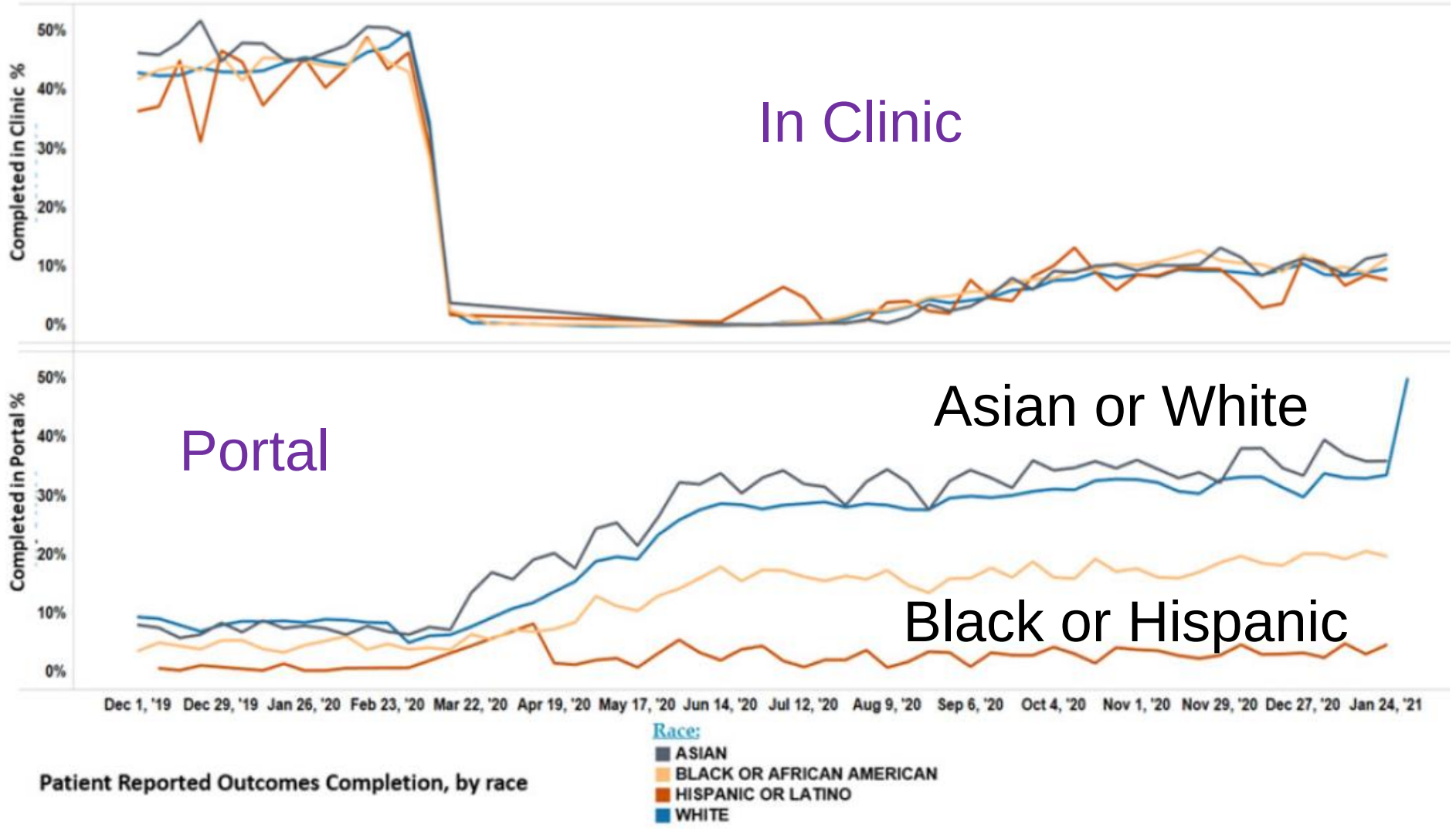


Background

- Patient-reported outcomes (PROs) provide **essential safety and tolerability data** to inform patient-centred clinical care and regulatory decisions
- Research using PROs often fail to address cultural and health specificities of populations underserved by research[3,4]
- Some groups may not benefit from PRO data
- If groups are systematically excluded, **health data poverty** occurs[5], omitting vital evidence relating to these groups when informing clinical care, regulatory decisions, and health policy[3].

Examples of barriers to PRO completion





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Sisodia RC, et al. JAMIA. 2021;28(10):2265-2268.

Underserved groups in PRO research

Slade *et al. Trials* (2021) 22:306
https://doi.org/10.1186/s13063-021-05255-z

Trials

REVIEW Open Access

Systematic review of the use of translated patient-reported outcome measures in cancer trials

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Abstract

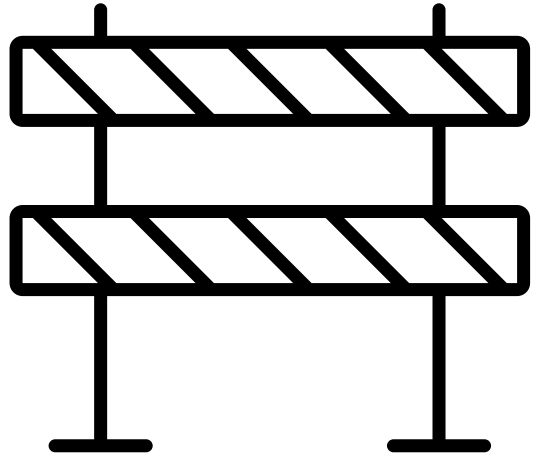
Background: Patient-reported outcomes (PROs) are used in clinical trials to assess the effectiveness and tolerability of interventions. Inclusion of participants from different ethnic backgrounds is essential for generalisability of cancer

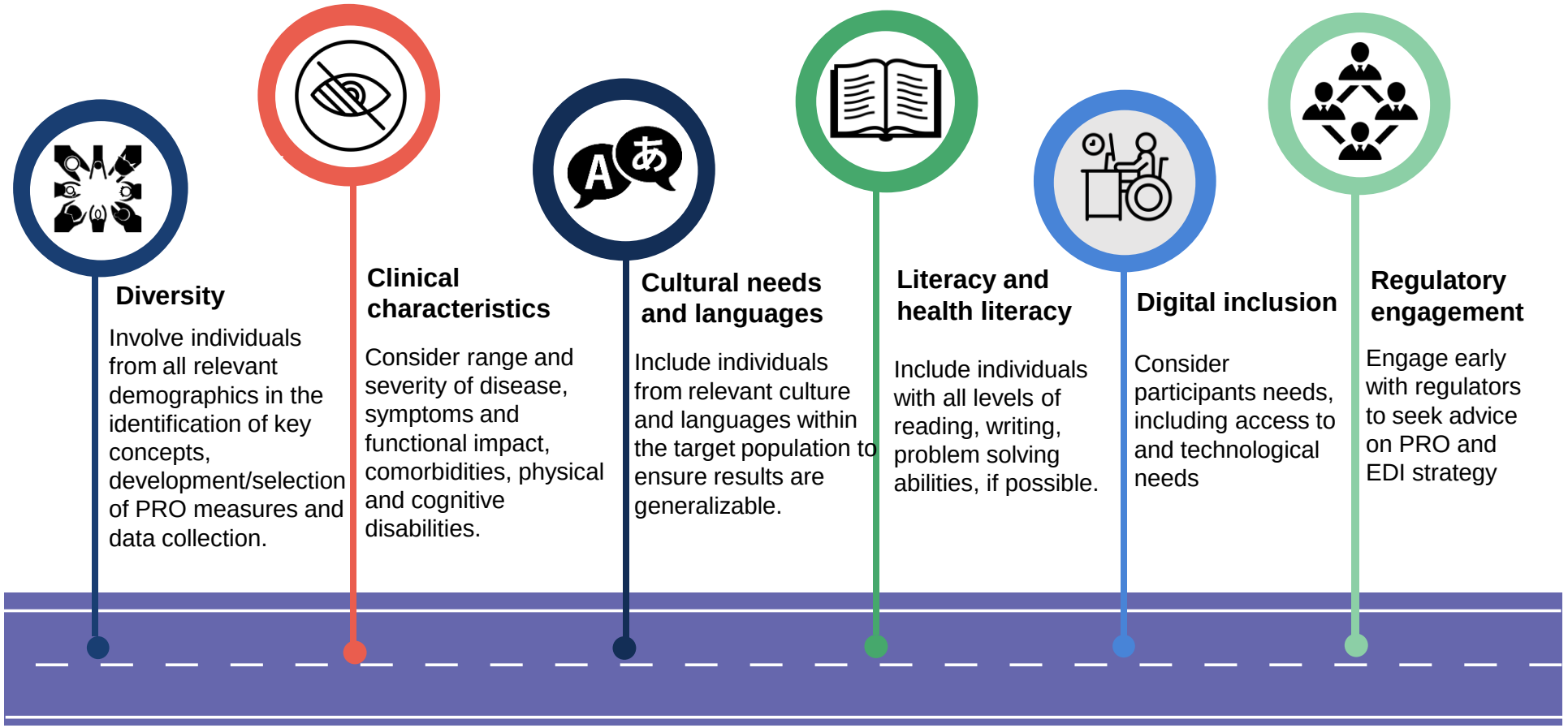
Key findings

- 84 cancer clinical trials on NIHR portfolio using PRO endpoint
- 14 (17%, n=4754) reported ethnic group data
- 8 multicentred and multinational, none reported translated PROMs though available for 7 of these studies
- Perceived barriers – difficulty engaging, relevance of ethnicity to research question, prominence of PRO in overall trial, investigator burden
- Community engagement at an early stage

Why does this happen?

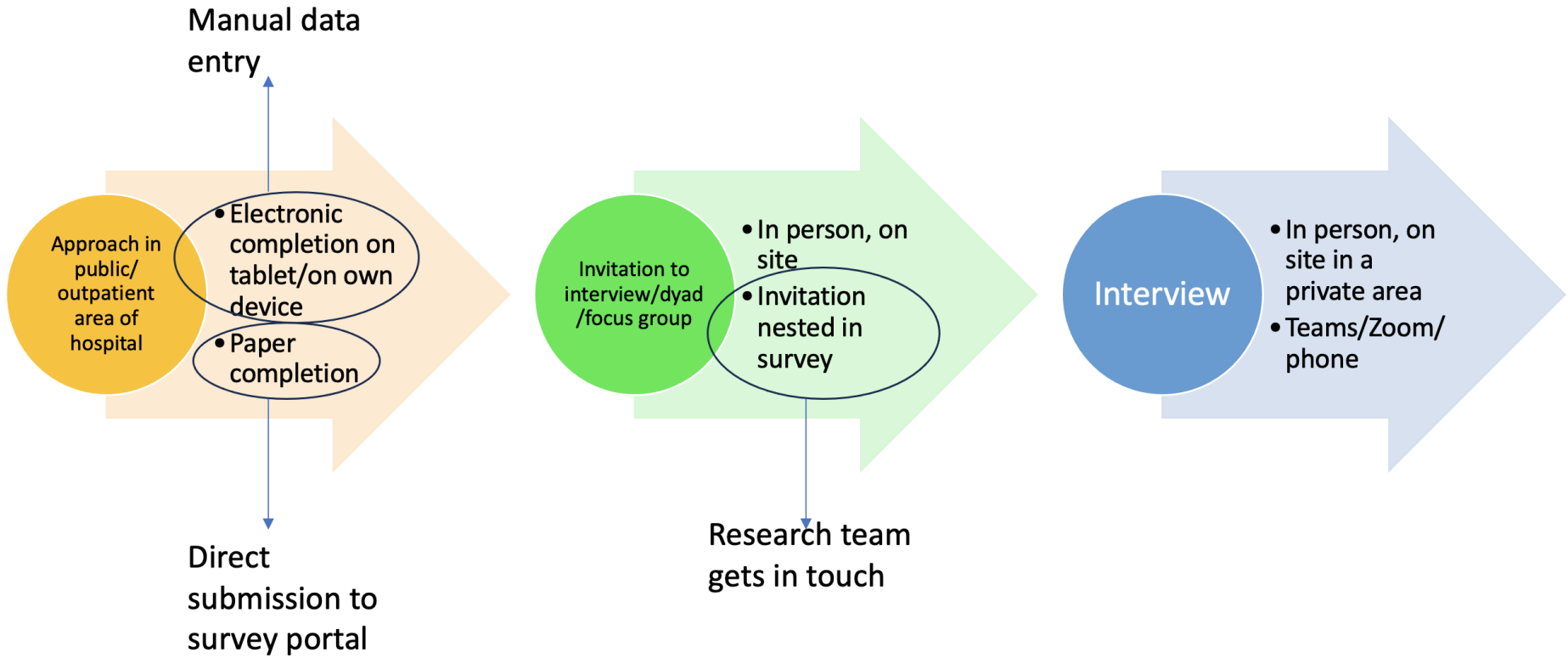
- Narrow eligibility criteria
- Reliance on recruitment strategies that work for only certain groups
- Failure to recognise historical research legacy
- Poor engagement and retention of participants
- Preconceptions around cost of innovative recruitment strategies
- Perceived cost of involvement and inclusion




Diversity
Involve individuals from all relevant demographics in the identification of key concepts, development/selection of PRO measures and data collection.
Clinical characteristics
Consider range and severity of disease, symptoms and functional impact, comorbidities, physical and cognitive disabilities.
Cultural needs and languages
Include individuals from relevant culture and languages within the target population to ensure results are generalizable.
Literacy and health literacy
Include individuals with all levels of reading, writing, problem solving abilities, if possible.
Digital inclusion
Consider participants needs, including access to and technological needs
Regulatory engagement
Engage early with regulators to seek advice on PRO and EDI strategy

PROs and the Birmingham BRC

- World-leading centre focusing on **inflammation**.
- More than 50% of deaths due to long-term inflammation-related diseases - **major NHS and global priority**[8].
- 5.7 million people, **socially diverse, multi-ethnic, significant health inequalities and life expectancy lower** than the UK average[9].
- Groups underrepresented in research are **willing to participate**, but inclusive research to explore public attitudes towards PRO research specifically is limited.
- Factors linked with being underserved by research are **also associated with inflammation**[10-12],
- Low **socio-economic status interacts with inflammation** throughout the life-course[13-14].
- Inclusive PRO strategies for benefits and risks of PRO research are to be **equitably distributed**
- PRO strategies must be **inclusive** of those from whom these data are sought and aim to serve.


Manual data entry
Approach in public/ outpatient area of hospital
• Electronic completion on tablet/on own device
• Paper completion
Direct submission to survey portal
Invitation to interview/dyad /focus group
• In person, on site
• Invitation nested in survey
Research team gets in touch
Interview
• In person, on site in a private area
• Teams/Zoom/ phone

Research Inclusion

- **Diverse NHS trusts** to capture a broad and representative sample.
- Minimise **digital exclusion**, offering the survey in paper and electronic forms (on own device/provided device) with assistance readily available, using in-person recruitment.
- Interviews offered using **video-conferencing software and telephone**.
- **Options for provision of personal data** e.g. not requiring contact information or names.
- Prespecified **multiple regression analysis** with participants' demographic and socio-economic survey data.
- Qualitative participant sampling to achieve **maximum variation** according to participants' demographic and socio-economic characteristics.
- **Participants not be excluded** due to demographic and socio-economic characteristics.
- Participant characteristics collected based on **relevance to the research aims**
- Oversight from the patient and public involvement panel - **acceptability and data minimisation.**

Lessons learned

- Difficult to recruit
- Language
- Operational issues
- Tricky consent procedures
- Paper versus electronic completion
- Pilot phase

Results and Impact

- Where sample size allows, parallel exploratory analyses will be conducted.
- Thematic analysis of qualitative data will be presented.
- Findings will be interpreted in partnership with public contributors.
- This study will generate guidance to reduce potential health inequalities perpetuated by PRO implementation.

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